Application Number: T3-00002 **Scientific Score**: 60 or below

Title: CIRM Training Program: Stem Cell Based Therapy of Parkinson's Disease

Specific names of individuals and institutions are blacked out to preserve applicant confidentiality where possible.

Proposal Abstract as Submitted by Applicant

The purpose of this program is to provide a Specialized Training Program (Type III) in research and clinical application of stem cell technology to the treatment of Parkinson's disease and related disorders. The program will support six post-doctoral Scholars drawn from the clinical and basic sciences. The translational nature of the program is emphasized by a disease-oriented curriculum of formal training and research and by faculty of experienced basic researchers and clinician scientists. Mandatory training will involve coursework on stem cell biology and regenerative medicine, and on Parkinson's disease research and clinical principles. Other training activities will include attendance at seminars, scientific retreats and national and international meetings and elective rotations through the laboratories of different faculty. [redacted] is a world-recognized institution for bench-to-bedside research into new treatments for Parkinson's disease. Experimental models of the disease, ranging from in vitro neuronal cultures to in vivo animal systems (rodents, non-human primates), will be available for studies on the neuroregenerative effects of stem cells against nigrostriatal degeneration. Clinical fellows interested in stem cell therapy will also have the opportunity to learn about the design of clinical trials and to develop their proficiency in patient evaluation. The program will be based at [redacted], but will take advantage of collaborative activities that are already in place and will be further developed between [redacted] and [redacted]. The administrative organization of the program will consist of the program Director ([redacted]) and Adjunct Director ([redacted]), a Steering Committee and an External Review Committee. A primary function of these committees will be to assess the quality of training and research and to ensure that CIRM Scholars reach their educational and career goals related to stem cell-based treatment of Parkinson's disease.

Benefit of this Program to California

This program will benefit the people and the state of California by providing high-quality training in the scientific, clinical, social, and ethical aspects of stem cell research to the scientists and clinicians who will develop and apply future therapies in this rapidly emerging field.

Summary of Review

This application proposes a highly focused type III program intended to train four post-doctoral and 2 clinical trainees. Scholars will be immersed in both basic research and clinical training in stem cell applications to Parkinson's disease. The institution offers outstanding capabilities in both experimental models and clinical treatment of Parkinson's disease, which reviewers consider an important target for stem cell therapies. The applicant institution intends to share training responsibilities, particularly course work and resources, with a large and well-regarded university. At the time of this

application, arrangements were not yet finalized for joint training at the university. The program director is an expert in animal models of Parkinson's disease and serves as director of basic research for the institution. The research faculty available in-house for mentoring is small (only six members) but has considerable expertise in cell-based therapies for Parkinson's disease. Expertise is not in stem cell research, per se. Therefore, the request for six trainees with only five or six available mentors seems high. The application did not provide CVs for the program director or faculty, and therefore it is difficult to evaluate their capabilities as mentors. A further weakness of the application is the lack of documentation about past trainees and existing training programs, which makes evaluation of this program difficult. Trainees will be drawn from the current post-doctoral and clinical fellows at the institution. This institution has great expertise in Parkinson's disease, but it does not explain how the program plans to train CIRM scholars.

Overall Strengths and Weaknesses

The strength of this application is the focus on a disease that may be an important early application of stem cell therapeutics. A possible collaboration with a large university is considered a strong point that would enhance training capability, but arrangements appear to be at an early stage. The major weaknesses of the application are the lack of documentation about past trainees, current training programs, faculty research interests, and mentoring success.

Recommendations

Not recommended for funding at this time.

	Pre	Post	Clinical	Total
Fellows Requested:	0	4	2	6
Fellows Recommended:	0	0	0	0

	Year 1	Total
Budget Requested:	$ 496,650	$ 1,489,950
Budget Recommended:	0	0